

**OBJECTIVES:** Colorectal cancer (CRR) is an important public health problem. The human and financial costs of this disease have prompted considerable research efforts to evaluate the ability of screening tests to detect the colorectal cancer at an early curable stage. It is now established that screening by faecal occult blood test (FOBT) in average-risk populations can detect asymptomatic colorectal cancers and precancerous lesions (high-risk adenomas). **METHODS:** We have reviewed the evidence about the quality, accessibility, and cost of screening using the FOBT to reduce colorectal cancer mortality. Such databases as Medline, PubMed, EMBASE were used. **RESULTS:** Faecal occult blood test screening benefits include reduction in CRR mortality, possible reduction in cancer incidence through detection and removal of colorectal adenomas and potentially, treatment of early colorectal cancers may involve less invasive surgery. Thus, implementation of biennial faecal occult blood test screening is an efficient use of health resources. **CONCLUSIONS:** The main objective for the improvement of cancer patients' care is the introduction of national colorectal cancer screening programs based on evidence and available to people across the country. Introduction of clinical and cost-effectiveness screening methods in Ukraine and coordination of Ukrainian and world practices will provide timely and effective medical care for patients with CRR. In order to integrate these approaches into clinical practice based on evidence the clinical protocols are being developed.

## PCN213

## A COMPREHENSIVE ASSESSMENT OF EARLY TRIAL EVIDENCE IN PRIMARY BREAST CANCER: HOW DECISIONS CHANGE OVER TIME

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**BACKGROUND:** There is ongoing debate over the extension of accelerated approval and conditional market authorisation initiatives, particularly for new cancer drugs in the US and Europe. This raises concerns for the benefit-risk assessments of both regulators and payers when relying on early trial evidence. Methodological issues relating to immature clinical data and short-term follow-up, as well as the potential use of surrogate endpoints, may undermine these assessments and the resulting decisions made under conditions of uncertainty. In the context of health technology assessments (HTA) in the UK, we considered the impact on the probabilities of being the most effective and cost-effective treatment when reviewing available evidence along the drug development and assessment processes. **OBJECTIVES:** To conduct a retrospective analysis of four NICE technology appraisals in primary breast cancer for HER-2 positive women alongside a simulation study to evaluate the relative effectiveness and cost-effectiveness of selected drugs over time. **METHODS:** We extracted data from TA107, TA108, TA109 and TA112 to analyse 'snapshots' of evidence at different time-points. Based on this example, we simulated individual patient characteristics and progression-free survival (PFS) times according to a predefined recruitment timeline. For both applied and simulated datasets, we combined a network-meta-analysis and three-stage Markov economic model in WinBUGS to predict the joint impact of growing evidence networks on HTA results. The model allowed for different assumptions regarding the extrapolation of and the correlation between PFS and overall survival. **RESULTS:** The magnitudes of credible intervals depended heavily upon the level of restriction used for follow-up. In addition, the performance of PFS as a surrogate endpoint strongly relied on the assumptions made on the correlation between the two endpoints. **CONCLUSIONS:** Our analysis highlighted the inherent limitations and risks of early assessments with a substantial increase in the uncertainty of treatment acceptability at thresholds of £20K and £30K.

## PCN214

## EXPERT ELICITATION USED FOR EARLY TECHNOLOGY ASSESSMENT TO INFORM ON COST-EFFECTIVENESS OF NEXT GENERATION SEQUENCING

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**OBJECTIVES:** Next Generation Sequencing (NGS) promises to find mutations (targets) in individual cancer patients, to subsequently assign targeted therapy. Currently, the technology itself is still in development and the effects on disease development, prognosis, or choice of therapy are still unclear. Besides information for the current patient, additional (secondary) information for future patients can also become available. To accelerate the reimbursement process and have a NGS-panel available for patients in the earliest possible stage, early Technology Assessment (TA) is ongoing. In this project, we report on expert elicitation by means of scenario drafting, to provide qualitative and quantitative data to fill the evidence gaps in early cost-effectiveness modeling. **METHODS:** The following steps in a multi-parameter framework can be distinguished: 1) Identifying (dynamic) aspects having impact on adoption; 2) Brainstorm on possible scenarios (informal interviews with NGS experts); 3) Scenario construction; 4) Validation of scenarios (semi-structured questionnaires to European NGS-experts); 5) Quantification into parameters for cost-effectiveness modeling. **RESULTS:** Based on the interviews and questionnaires (n=29), the most likely scenarios as patients interest in NGS (likelihood 66.5% ±28.1); organizational readiness (84.4 ±18.5); advantage of including RNA, and the demand from the clinic (needs of medical staff) were identified as drivers for NGS development. Possible barrier scenarios were: number of actionable targets (55.2 ±23.7); demonstrating clinical utility (50.4 ±31.4); current evidence generation (65.5 ±27.9); consensus on panel for reimbursement (40.3% ±24.1); and competition within the field (64.2 ±21.9). Clustering into parameters for cost-effectiveness modeling resulted in: "failures", "compliance", "uptake", and "future information". **CONCLUSIONS:** Although there are many issues to overcome to adopt NGS, the likelihood of NGS incorporation in clinical practice is very high. The "additional future information" is the most interesting but complex variable to identify and to incorporate in cost-effectiveness modeling.

## PCN215

## NEXT GENERATION SEQUENCING TECHNOLOGY: HEALTH TECHNOLOGY ASSESSMENT, MARKET ACCESS TRENDS AND POTENTIAL IMPACTS ON THE FUTURE OF COMPANION DIAGNOSTIC TESTING

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**OBJECTIVES:** Next Generation Sequencing (NGS) offers a potentially powerful platform for extremely sensitive, high-throughput, multiplex, quantitative detection of nucleic acid biomarkers. While NGS currently represents a small portion of global clinical molecular diagnostic testing, new funding and reimbursement initiatives promise to accelerate its clinical utilization. Given increasing numbers of predictive/ prognostic biomarkers but limited tissue and need for less invasive sample acquisition, NGS has the potential to transform personalized medicine (PM) and companion diagnostics. The current study characterized global NGS availability and reimbursement trends. Health technology assessments (HTAs) for NGS and other relevant multiplex/ gene panel tests were also studied for evolving evidence requirements. **METHODS:** Key health care provision, HTA agency, and payer websites in the EU, US, Australia and Canada were reviewed to identify NGS funding and reimbursement initiatives, and relevant HTAs. In addition, a limited number of stakeholder interviews were conducted to help further characterize the evolving global NGS landscape. **RESULTS:** A number of NGS funding and reimbursement initiatives were identified, especially France, Germany, UK, US and Australia. Initiatives have been mainly centered on funding of pilot clinical utility demonstrations through research and clinical use. In Germany and US, specific initiatives are underway to develop specific NGS reimbursement codes and payment rates. A number of HTAs for NGS and other multiplex/ gene panel test platforms were identified, primarily for oncology, cardiovascular, infectious disease, inherited disease, and neuropsychiatry applications. Key HTA concerns include test clinical utility, cost-effectiveness, real-world reproducibility and equity of access given potential cost. **CONCLUSIONS:** Payers and providers increasingly recognize NGS as enabling expanded adoption of PM approaches. As PM expands with increasing numbers of clinically actionable biomarkers, ensuring that test evidence development is aligned with expectations, and expectations with reality are key steps. Further, developing reimbursement/ funding mechanisms to support testing uptake will be critical in all markets.

## PCN216

## HOW IS RESEARCH AND DEVELOPMENT INNOVATION EVOLVING? FOCUS ON ONCOLOGY AND CARDIOVASCULAR DISEASE

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By 2030, 50% of all deaths are expected to be caused by oncology, or cardiovascular diseases. Research and development therefore continues to evolve in these areas to provide a better answer to complex medical needs. As a consequence, the definition of innovation is evolving, going from therapeutic drugs with a new mode of action to novel biotechnologies, biological therapeutics, and vaccination. **OBJECTIVES:** We aim to investigate how therapies for oncology and cardiovascular diseases are expected to evolve in future into technologies that transform the concept of innovation. **METHODS:** Data were extracted from Citeline to assess the scale of therapies in development including: vaccines, stem cells, antibodies, reformulation of combination therapy, gene therapy and RNAi. **RESULTS:** In oncology, across the selected novel therapies, there are over 670 trials in preclinical to phase 3 of development. Vaccines have a higher rate of development with over 200 products in preclinical development to phase 3. Other areas of development include antibodies, and gene therapy, with over 30 therapies in phase 2 trials alone. Meanwhile, cardiovascular disease has fewer ongoing 'novel therapy' trials compared to oncology (around 75% fewer trials). The main areas of development include reformulation of fixed dose combinations, and stem cell research (a total of 60 trials, and 53 trials in preclinical to phase 3 respectively). The trend in innovation for cardiovascular disease instead tends to be focussed on integrated technology and medical devices. **CONCLUSIONS:** While we are fairly acquainted with the current evaluation methods and relevance of clinical and economic evidence of traditional therapies, the emergence of new technologies creates uncertainties around how they will be assessed by payers. For example, demonstrating the value of vaccines (which avoid illness) is difficult to express. Industry must now consider factors outside of the current remit to prepare for successful market access.

## PCN217

## 5-YEAR SURVIVAL IS NOT AN APPROPRIATE INDICATOR FOR CANCER CONTROL IN THE POPULATION: REVISITING THE ISSUE BASED ON UK DATA

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**OBJECTIVES:** It is controversial to use 5-year survival estimates to assess the progress in cancer prevention and control. The study aimed to analyze associations between 5-year survival, and two standard measures of cancer burden, incidence and mortality, based on publicly available population cancer statistics in England and Wales. **METHODS:** Sex-specific mortality and incidence of 14 types of common cancer between 1976 to 1995 were obtained from a national database on the UK Office for National Statistics website. Sex-specific 5-year survival data retrieved from the Cancer Research UK website databases. The relationships between 5-year survival, and incidence and mortality were estimated based on both Pearson and Spearman correlation coefficients. **RESULTS:** From 1976 to 1995, all male and female cancer types showed increased 5-year survival, ranged from 0.2% (pancreas and lung cancers) to 16.6% (prostate cancer) for males, and from